Stem Cell Agency Funds Three New Clinical Trials at Stanford

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Oakland, CA – Today the governing Board of the California Institute for Regenerative Medicine (CIRM) awarded $30.98 million to Stanford University to fund three clinical trials. This brings the total number of CIRM-funded clinical trials to 75.

$11,998,988 was awarded to conduct a clinical trial to test a therapy for motor disabilities caused by chronic ischemic stroke. While "clot busting" therapies can treat strokes immediately after they occur (acute strokes), these treatments can only be given within a few hours of the initial injury. There are no approved drugs to treat the disabilities that remain in the months and years after stroke (chronic stroke).

Gary Steinberg, M.D., Ph.D., and his team at Stanford University will use neural stem cells (NSCs), a kind of stem cell that can form different cell types found in the brain. The NSCs, in turn, are derived from human embryonic stem cells (hESCs) and these can form virtually any human cell type. In a surgical procedure, the team will inject the NSCs directly into the brains of chronic stroke patients. While the ultimate goal of the therapy is to restore loss of movement in patients, this is just the first step in clinical trials for the therapy. This first-in- human trial will evaluate the therapy for safety and feasibility and look for signs of efficacy in the form of improved movement.

$11,998,310 was also awarded to conduct a clinical trial to test a treatment for children and young adults with glioma, a devastating, aggressive brain tumor that occurs primarily in children and young adults and originates in the brain. Such tumors are uniformly fatal and are the leading cause of childhood brain tumor-related death. Radiation therapy is a current treatment option, but it only extends survival by a few months.

Crystal Mackall, M.D., and her team at Stanford University will modify a patient's own T cells, an immune system cell that can destroy foreign or abnormal cells. The T cells will be modified with a protein called chimeric antigen receptor (CAR), which will give the newly created CAR-T cells the ability to identify and destroy the brain tumor cells. The CAR-T cells will be re-introduced back into patients and the therapy will be evaluated for safety and efficacy.

$6,987,507 was awarded to a third clinical trial to test a therapy for left-sided heart failure resulting from a heart attack. The major issue with this disease is that after a large number of heart muscle cells are killed or damaged by a heart attack, the adult heart has little ability to repair or replace these cells. Thus, rather than being able to replenish its supply of muscle cells, the heart forms a scar that can ultimately cause it to fail.

Joseph Wu, M.D., Ph.D., and his team at Stanford University will use hESCs to generate cardiomyocytes (CM), a type of cell that makes up the heart muscle. The newly created hESC-CMs will then be administered to patients at the site of the heart muscle damage in a first-in-human trial. This initial trial will evaluate the safety and feasibility of the therapy, and the effect upon heart function will also be examined. The ultimate aim of this approach is to improve heart function for patients suffering from heart failure.

"We are pleased to add these clinical trials to CIRM's portfolio," says Maria T. Millan, M.D., President and CEO of CIRM. "Because of the reauthorization of CIRM under Proposition 14, we have now directly funded 75 clinical trials. The three grants approved today bring forward regenerative medicine clinical trials for brain tumors, stroke, and heart failure, debilitating and fatal conditions where there are currently no definitive therapies or cures."

An additional $12.85 million was awarded to 11 recipients for Discovery Stage Research Projects (DISC2), which promote the discovery of promising new stem cell-based and gene therapy technologies that could be translated to enable broad use and ultimately, improve patient care.

The awards are summarized in the table below.

Title	Institution	Award Amount

Stem cell-derived extracellular vesicles to reverse radiation-induced brain injury	UC Irvine	$1,064,724
A universally applicable skin sheet for Dystrophic Epidermolysis Bullosa via next-generation gene editing, iPS cell technology and tissue engineering	Stanford	$1,420,200
Human iPSC-derived chimeric antigen receptor expressing macrophages for improved cancer treatment.	UC San Diego	$1,414,917
AAV9-Cas13 gene therapy for Angelman syndrome	UC Davis	$1,364,903
Development of a new therapeutic for directing target specific stem cell migration and treatment	UC San Diego	$1,129,512
Hypoxia-specific Production of Exosomes from iPSC-derivatives for Myocardial Repair	Stanford	$1,418,023
A novel hybrid CRISPR tool for gene network perturbation and hiPSC engineering	Stanford	$705,733
Targeting Critical Regulators of Cancer Stem Cells	UC San Diego	$1,257,814

Small Molecules to inhibit Nemo-like Kinase for Treatment of Diamond Blackfan Anemia	Stanford	$848,098
Building a hiPSC-based biopacemaker	UC Davis	$1,414,113
iPSCs as a screening tool to predict risk of nonalcoholic fatty liver disease	UC San Francisco	$813,000

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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